Australian Government
Department of Health
Therapeutic Goods Administration

Evolving role of Real-World Evidence in supporting access to medicines

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TGA Health Safety Regulation

Why now

- Consultation in 2021 revealed industry and consumers are unclear on how the TGA uses Real World Evidence (RWE)
- Increasing availability of RWE
- Opportunities for improved treatments and repurposing of medicines
- Clinical trial evidence versus RWE in applications

What is the TGA doing?

Opening Dialogue

Setting definitions and guidelines

Increasing our transparency on RWE

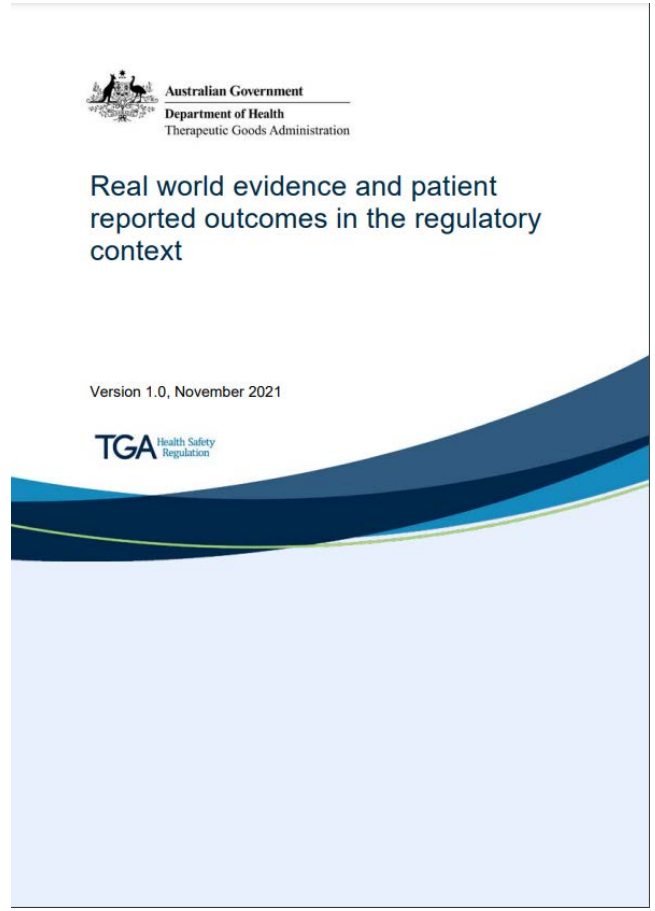
Increase communication and understanding of RWE amongst internal and external stakeholders

Rapid Review

May 2021

Rapid review commissioned by the TGA

- Examined COR regulatory documents on RWE
- Performed 50 targeted stakeholder interviews
- Review found:
  - Ambiguity (internally and externally) surrounding TGA's RWE use, potentially limiting its adoption.
  - Recommended TGA improve communication about how we accept and use RWE.



November 2021

Web statement and report published
https://www.tga.gov.au/review-real-world-evidence-and-patient-reported-outcomes

TGA response 2021 – 2022

- Establish a central point for RWE information on TGA website
- Develop Australian RWE definition
- Collaborate with overseas regulators
- Consult on the development and adoption of guidance documents
- Provide guidance around use of RWE for pre-market evaluations
- Communicate when RWE has been used to make a regulatory decision
- Support RWE use in Orphan, Provisional and Repurposing of medicines

Sponsors declare RWE usage in dossiers	→	eCTD Module 1
TGA transparency around use of RWE in pre-market evaluation of medicines	→	AusPARs
TGA to consult on guidance around usage and evaluation of RWE in premarket submissions	→	International and Australia specific guidance adoption
Review implications of RWE use in particular for Orphan or Provisional pathways and for Repurposing of medicines.	→	Provide support and guidance
RWE presence on TGA website	→	RWE Web statement and Consultation summary published November 2021

The growing importance of RWE globally

Valuable data accumulated in many areas of healthcare

Difficult-to-treat disease use case data collection	FDA's CURE ID app allows health care providers to report new uses of existing drugs for 325 difficult-to-treat, infectious diseases through a standardised and simple case report form (CRF) from a website or mobile device
Populations excluded from clinical trials	Health Canada aims to leverage RWE to expand evidence-based indications for populations such as children, seniors and pregnant women
Orphan products conditions with unmet need	RWE may be feasible where RCTs are not, thus opening up the market for more treatments for patients with rare conditions on complex treatment pathways

RWE challenges

How do we ensure the scientific rigor of RWE?

Are potential biases identified and addressed through consistent data collection?

How does RWE meet the intended regulatory requirements?

How are health outcomes validated?

What is the RWE hierarchy of likely best evidence for given treatments?

How do we classify RWD and sample sizes?

What are “core” data elements, what are “optional” data elements?

Levels of evidence

Traditional levels of Evidence

https://www.researchgate.net/figure/Hierarchy-of-evidence-pyramid-The-pyramidal-shape-qualitatively-integrates-the-amount-of_fig1_311504831

Summary

- RWE offers opportunities for improved healthcare, including for rare and infectious disease treatment and the repurposing of medicines
- The reliability of the evidence is critical
- RWE is already being used and the TGA will enhance its application guidance and clarify its use in the decision making process
- The framework TGA adopts will align with those of overseas regulators
- The TGA will increase its transparency of RWE use in decision-making
- Next steps will be carried out in consultation with stakeholders

Q&A

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